

PROMS for studies in rheumatology

ASAS health index as an example

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Potential conflicts of interest

- Current chair of EULAR's committee for the Quality of Care
- Past chair and current executive committee member of ASAS
- Director of Rheumatology Consultancy bv
- Consultant for AbbVie, BMS, Eli-Lilly, Galapagos, Gilead, Jansen pharma, Novartis UCB-pharma,

EULAR: European League against Rheumatism

ASAS: Assessment in Spondyloarthritis International Society²

Rheumatology

- Chronic *inflammatory* diseases involving: joints, bone, spine, tendons, muscles, organs, etc
- Prototype is *rheumatoid arthritis*
- Structural damage (joint destruction, deformities)

Outcomes in rheumatology

Subjective information

- Pain, stiffness, fatigue, physical function impairment, quality of life

(More) objective information

- Joint swelling, imaging-inflammation, acute phase reaction, damage

>100 different (uni- and multidimensional) assessments (incl. PROM's)

Outcome measures development since 1992



- Development of core-domain sets
- Prioritisation of instruments



ORIGINAL ARTICLES

**Developing Core Outcome Measurement Sets for Clinical Trials:
OMERACT Filter 2.0[☆]**

Maarten Boers^{a,*}, John R. Kirwan^b, George Wells^c, Dorcas Beaton^d, Laure Gossec^e,
Maria-Antonietta d'Agostino^f, Philip G. Conaghan^g, Clifton O. Bingham III^h, Peter Brooksⁱ,
Robert Landewé^j, Lyn March^{k,l}, Lee S. Simon^m, Jasvinder A. Singh^{n,o}, Vibeke Strand^p,
Peter Tugwell^q

clinicians

patients

researchers

pharma

regulators

Truth – Discrimination – Feasibility

the OMERACT filter

1. TRUTH

*Is the measure truthful? Does it measure what is intended? Is the result unbiased?
(face, construct, content)*

2. DISCRIMINATION

*Does the measure discriminate between states of interest?
(sensitivity to change, reliability)*

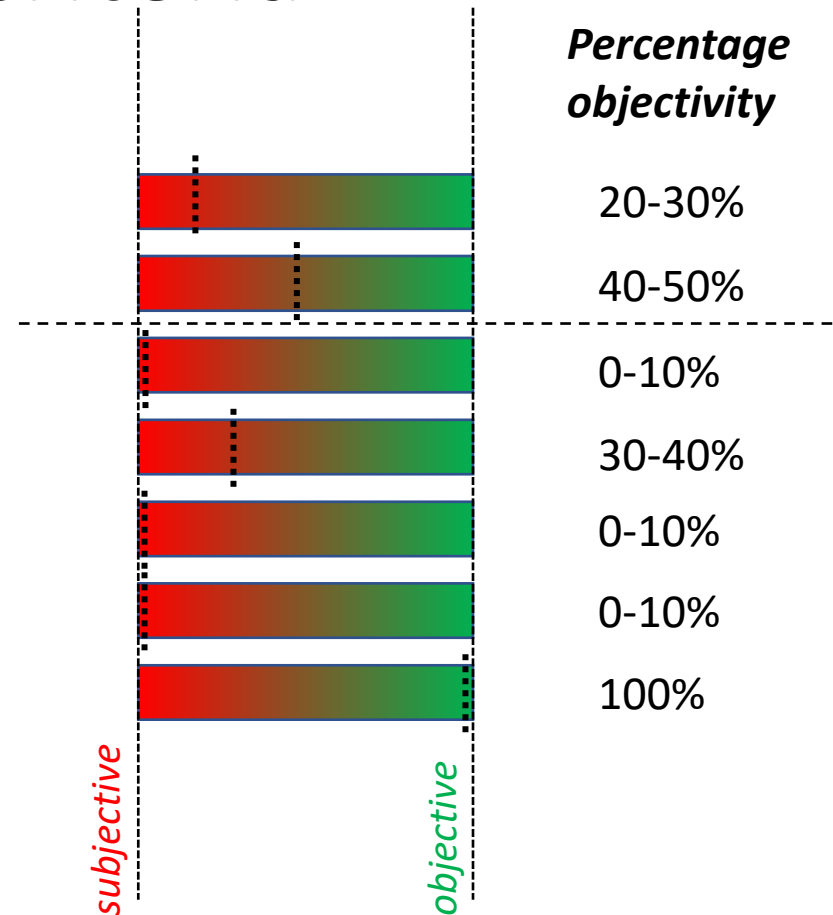
3. FEASIBILITY

*Can the measure be applied easily
(time, money, interpretability)*

ACR 20-50-70 response criteria

rheumatoid arthritis

- Tender joint count
- Swollen joint count
- Patient global assessment of disease activity
- Physician global assessment of disease activity
- Pain
- HAQ-score
- CRP

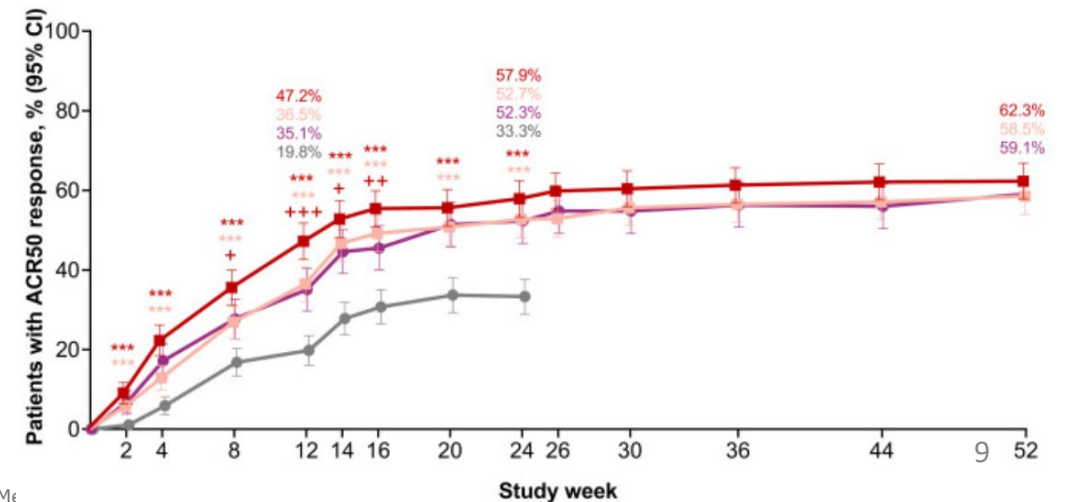
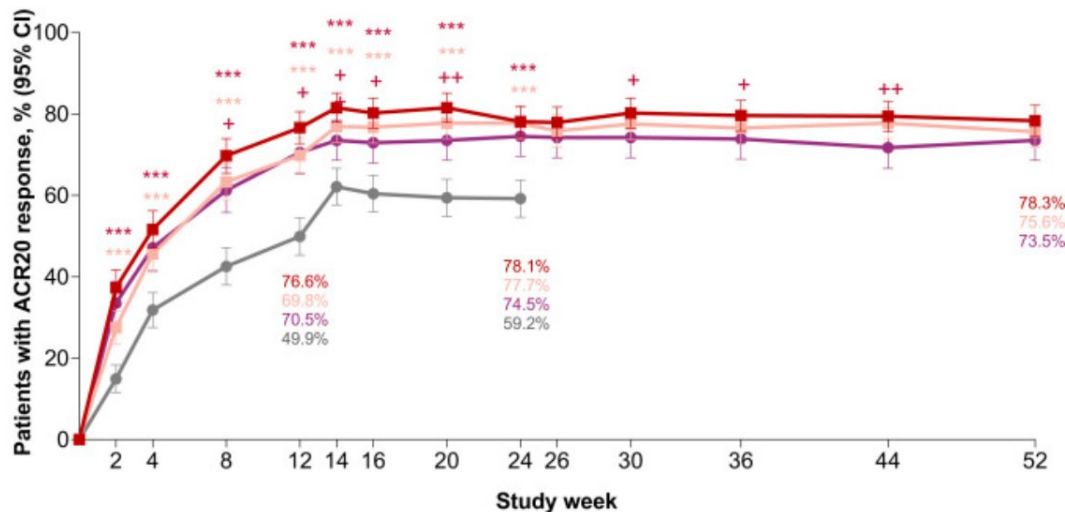


Patient-reported | Assessor-reported | Independently measured

Stem: 65% subjective
Tail: 70% subjective

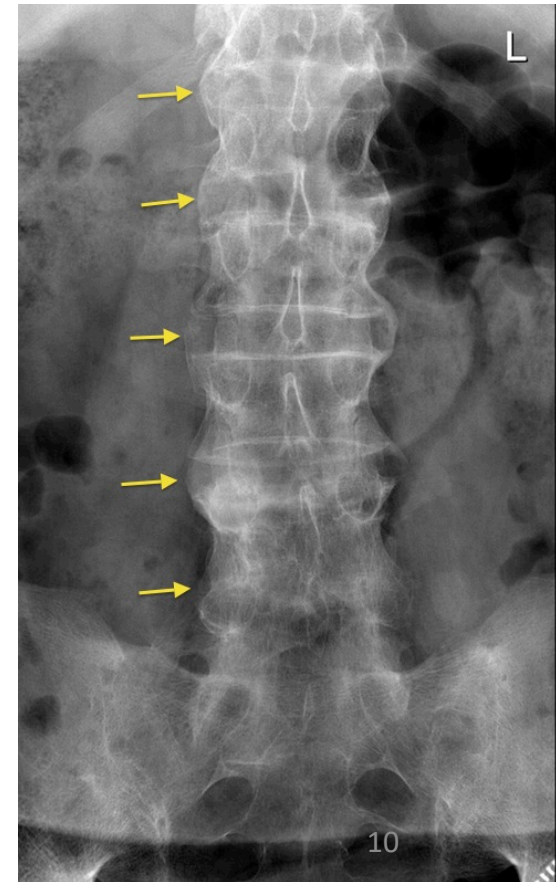
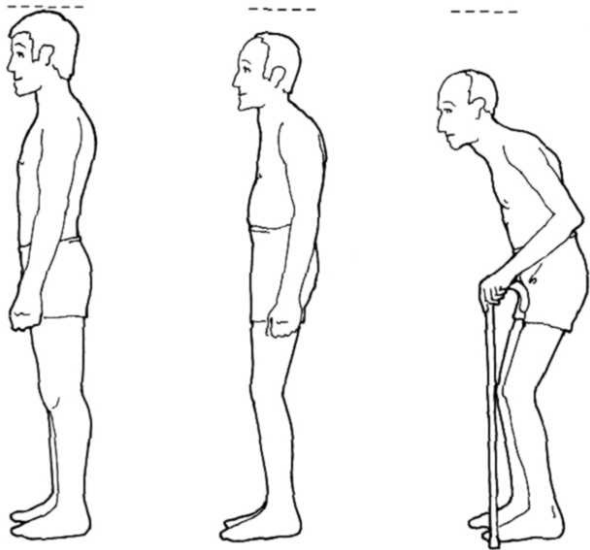
ACR 20-50-70 response criteria

- Developed by professionals
- Validated and endorsed by OMERACT as core-set measures
- Approved by regulatory agencies across the world as primary endpoint in Phase 2/3 RCTs
- Comparability across trials during the recent decades



Axial Spondyloarthritis (axSpA)

- Previously Bechterew's disease
- Debut in young adolescents
- Chronic inflammation of the spine
- 'Bamboo spine' in 10%; bone spurs (syndesmophytes) in 30%



Outcomes

DIRECT

- Inflammation causes pain, stiffness, fatigue
- Functional impairment

LATER

- Stiff spine
- Co-morbidities
- Job loss
- Interference with Quality of Life



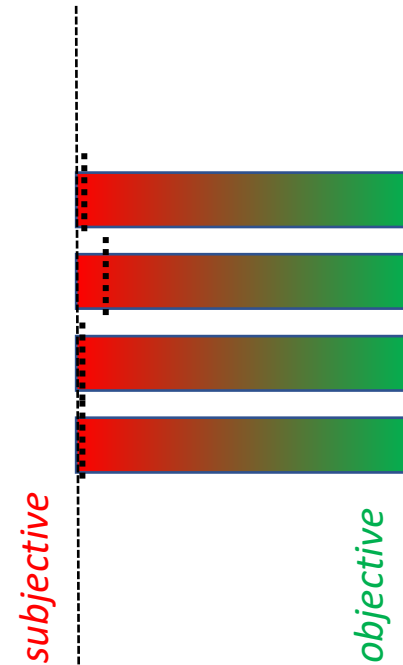
- An OMERACT look-alike (1995)
- Developing and updating a core-domain set for axial spondyloarthritis
- Prioritising instruments to assess these domains in studies

Axial spondyloarthritis

ASAS40 response

Components

- Pain
- Function (BASFI)
- Patient global assessment of disease activity
- 'Inflammation' (two NR-Scales)



90-100% patient-reported

axSpA and PRO's

- Symptoms assessed with PROM's
- Physical function assessed with PROM's
- Quality of Life (generic & disease-specific) assessed as PRO's

>75% of outcomes is *patient-reported*

Ankylosing Spondylitis *Quality of Life*

ASQoL

- Disease-specific quality of life instrument
- Developed and validated by researchers from *Galen Research*
- Website: '*Trusted by health professionals, such as the EMA*'
- For profit organisation: users have to pay

ASAS/OMERACT:

- Professionals and patients should be in the lead
- Instruments should be free for use

ASAS health-index (2015)

International Classification of Functioning, Disability and Health (ICF)

WHO's framework for measuring *health* and *disability*

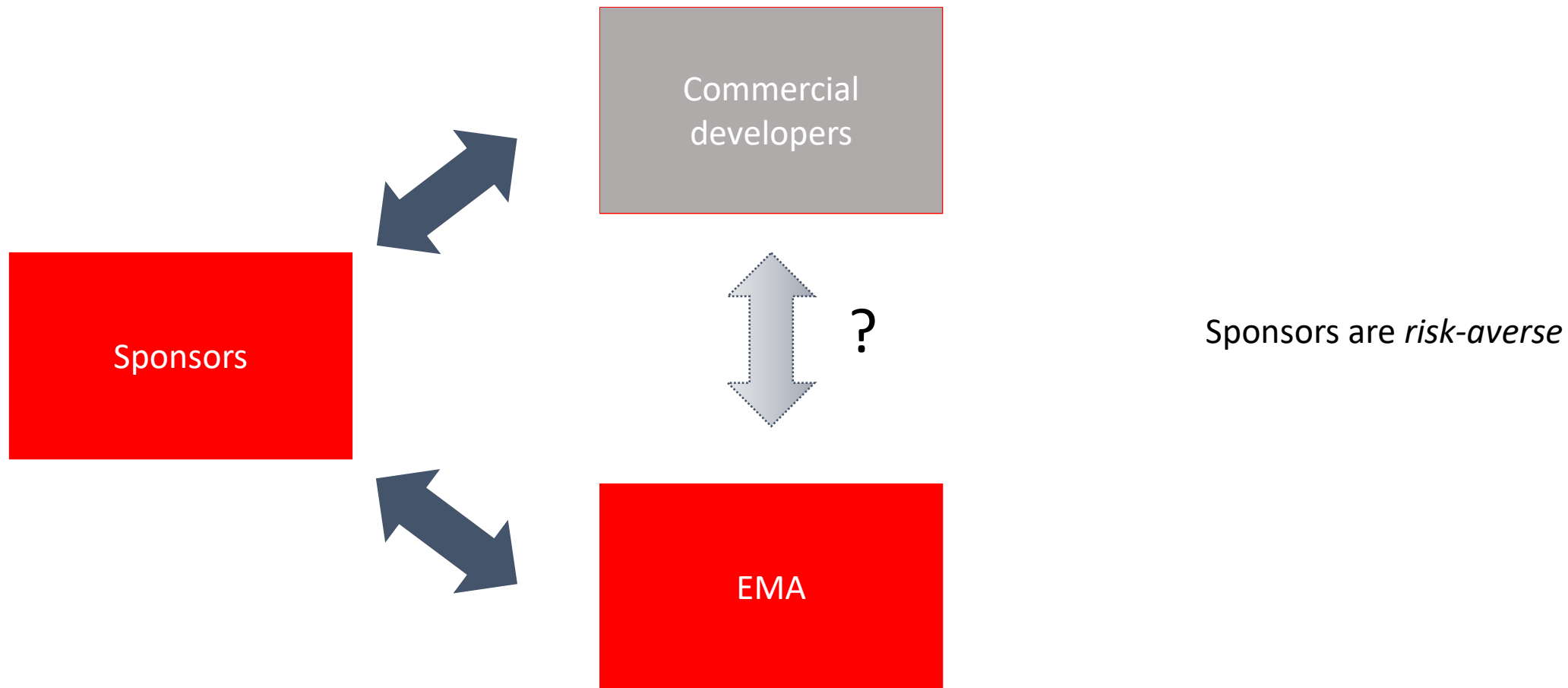


ASAS health index

- 17 items without redundancy (yes/no)
- Qualitative interviews
- Fundamental measurement principles
- PRO-mapping (preference-based)
- Translations validated
- etc



Use ASAS-HI in Phase 2/3 studies?



'Better' format for PROM-qualification

Tripartite consultation

